
Stem Cell Agency-Funded Clinical Trial for Age-Related Macular Degeneration Shows Encouraging Results

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Oakland, CA – A specially engineered patch that contains a sheet of stem cells has produced promising results in patients in an early phase clinical trial treating dry age-related macular degeneration (AMD), one of the leading causes of vision loss in the U.S. The trial is being funded by the California Institute for Regenerative Medicine (CIRM), the state's Stem Cell Agency.

Of the five patients enrolled in the Phase 1/2a trial, four maintained their vision in the treated eye, two showed improvement in the stability of their vision, and one patient had a 17-letter improvement in their vision on a reading chart. In addition, there were no serious side effects or unanticipated problems.

"Dry age-related macular degeneration is expected to affect around three million Americans by 2020, most of them over the age of 65, and right now there is no effective treatment," says Abba Creasey, Ph.D., Vice President of Therapeutics and Strategic Infrastructure at CIRM. "Having a therapy with a favorable safety profile, that could slow down the progression, or even reverse the vision loss would benefit millions of Americans. That's why these results, while still in an early stage are encouraging, because the people treated in the trial are ones most severely affected by the disease who have the least potential for visual recovery."

In AMD cells in the retina, the light-sensitive tissue at the back of the eye, are slowly destroyed affecting a person's central vision. It can make it difficult to do everyday activities such as reading or watching TV and make it impossible for a person to drive.

Researchers at the University of Southern California (USC) Roski Eye Institute at Keck Medicine, and Regenerative Patch Technologies, used embryonic stem cells that had been turned into retinal pigment epithelium (RPE) cells - the kind of cell destroyed by AMD. These cells were then placed on a synthetic scaffold that was designed to mimic Bruch's membrane, a key structure at the back of the retina that regulates the flow of oxygen to the eye and removes waste products. The scaffold containing the cells - called the California Project to Cure Blindness-Retinal Pigment Epithelium 1 (CPCB-RPE1) - was then surgically placed in the eye.

Imaging studies showed that the RPE cells appeared to integrate well into the eye and remained in place during follow-up tests 120 to 365 days after implantation.

There were other indications the implants were proving beneficial. People with normal vision have the ability to focus their gaze on a single location. People with advanced AMD lose that ability. In this trial two of the patients went from having unstable fixation to stable fixation. These improvements were maintained in follow-up tests.

"This is the first human trial of this novel stem cell-based implant, which is designed to replace a single-cell layer that degenerates in patients with dry age-related macular degeneration," says lead author and surgeon for the study, Amir H. Kashani, MD, PhD, assistant professor of clinical ophthalmology at the Keck School of Medicine of USC. "This implant has the potential to stop the progression of the disease or even improve patients' vision. Proving its safety in humans is the first step in accomplishing that goal."

"Our study shows that this unique stem cell-based retinal implant thus far is well-tolerated, and preliminary results suggest it may help people with advanced dry age-related macular degeneration," says coauthor and lead inventor of the implant Mark S. Humayun, MD, PhD, director of the USC Institute for Biomedical Therapeutics, co-director of the USC Roski Eye Institute.

While the results are encouraging the researchers caution that this was a very early stage clinical trial, with a small number of patients. They say the next step is to continue to follow the four patients treated in this trial to see if there are any further changes to their vision, and to conduct a larger trial.

"This project reflects CIRM's commitment to creating a pipeline of the most promising stem cell research and supporting it at every stage," says Dr. Creasey. "We began funding Dr. Humayun's research in 2010 and have worked closely with him and his team since then. We look forward to continuing our partnership and hopefully taking this encouraging start and making it even more successful in future clinical trials."

The study is published in the journal *Science Translational Medicine*.

About CIRM

At CIRM, we never forget that we were created by the people of California to accelerate stem cell treatments to patients with unmet medical needs, and act with a sense of urgency to succeed in that mission.

To meet this challenge, our team of highly trained and experienced professionals actively partners with both academia and industry in a hands-on, entrepreneurial environment to fast track the development of today's most promising stem cell technologies.

With \$3 billion in funding and approximately 300 active stem cell programs in our portfolio, CIRM is the world's largest institution dedicated to helping people by bringing the future of cellular medicine closer to reality.

About Regenerative Patch Technology LLC

Regenerative Patch Technologies LLC (RPT) is a California based company developing cell-based implant technology for the treatment of retinal diseases. CPCB-RPE1 is RPT's lead product. RPT was founded by Drs. Mark Humayun and David R. Hinton from the University of Southern California and Dr. Dennis O. Clegg from the University of California at Santa Barbara. The technology to produce the CPCB-RPE1 implant is exclusively licensed to RPT from the University of Southern California, the California Institute of Technology and the University of California at Santa Barbara. Further information about the company can be found at www.regenerativepatch.com.

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About the USC Roski Eye Institute

The USC Roski Eye Institute, part of the Keck Medicine of USC university-based medical enterprise, has been a leader in scientific research and innovative clinical treatments for more than 40 years. Ranked No. 2 in National Eye Institute (NEI) research grants for academically based ophthalmology departments and nationally ranked in *U.S. News & World Report's* annual "Best Hospitals" issue for more than 24 years, the USC Roski Eye Institute is headquartered in Los Angeles with clinics in Arcadia, Beverly Hills and Pasadena. Faculty physicians are also the exclusive ophthalmic doctors affiliated with Los Angeles County + USC Medical Center (LAC+USC) and Children's Hospital Los Angeles (CHLA).

For more information, go to eye.keckmedicine.org.

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